

# BioMarin to Highlight Breadth of Innovative Development Pipeline at R&D Day on October 18th in New York

SAN RAFAEL, Calif., Oct. 11, 2017 /PRNewswire/ --

Key program updates will include the following:



- BMN 270 gene therapy for hemophilia A: 4e13 dose and Phase 3 Study Design
- Vosoritide for achondroplasia: long-term extension data
- Pegvaliase for phenylketonuria: safety and efficacy data provided in BLA
- BMN 250 for MPSIIIB: GAG data and clinical data

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) will host an R&D Day from 8am (ET) to 1pm (ET) on Wednesday, October 18, 2017 in New York City. BioMarin management and external experts will provide an update to the investment community on the Company's development portfolio, which is focused on innovative therapies to treat rare and ultra-rare diseases.

External key opinion leaders scheduled to present include:

Maureen Cleary, M.D., F.R.C.P.C.H., Consultant in Inherited Metabolic Disease and Senior Lecturer at Institute of Child Health at Great Ormond Street Hospital for Children

- Clinical trial investigator on BMN 250 for MPS IIIB program

Nicola Longo, M.D., Ph.D., Professor of Pediatrics and Adjunct Professor of Pathology at the University of Utah

- Past President of the Society for the Study of Inborn Errors of Metabolism (SSIEM)
- Clinical trial investigator on pegvaliase program

Steven Pipe, M.D., Pediatric hematologist; Laurence A. Boxer Research Professor of Pediatrics and Communicable Disease, as well as Professor of Pathology at the University of Michigan

- Primary clinical interest is inherited and acquired bleeding and thrombotic disorders, including hemophilia, von Willebrand disease, rare coagulation factor deficiencies and thrombophilias

BioMarin speakers scheduled to present include:

Jean-Jacques Bienaimé, Chairman and Chief Executive Officer

Hank Fuchs, M.D., President, Worldwide Research & Development

Robert Baffi, Ph.D., Executive Vice President, Technical Operations

Geoff Nichol, M.B., Ch.B., M.B.A., Senior Vice President, Chief Medical Officer and Head of Global Clinical Development

David Jacoby, M.D., Ph.D., Executive Group Medical Director

Adam Shaywitz, M.D., Ph.D., Executive Medical Director, Clinical Science

Ben Kim, M.D., M.Phil., Senior Medical Director, BMN 270

Steve Maricich, M.D., Ph.D., Medical Director, Clinical Science

### **Live Video Webcast**

Interested parties may access a live video webcast that will include audio and slides of the presentations via the investor section of the BioMarin website, [www.biomarin.com](http://www.biomarin.com). A replay of the meeting will be archived on the site for at least one week.

For those who choose not to listen and view the event via webcast, dial-in information for the audio portion of the webcast can be accessed using:

U.S. / Canada Dial-in Number: (800) 319-9003

International Dial-in Number: (719) 325-3302

Conference ID: 138108

### **About BioMarin**

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.biomarin.com](http://www.biomarin.com). Information on BioMarin's website is not incorporated by reference into this press release.

#### Contacts:

##### Investors

*Traci McCarty*

*BioMarin Pharmaceutical Inc.*

*(415) 455-7558*

##### Media

*Debra Charlesworth*

*BioMarin Pharmaceutical Inc.*

*(415) 455-7451*

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